



Going beyond every day.™

August 19, 2026

Dear Members of the U.S. GSDIa Community,

We value the community's request for information and are committed to sharing timely updates. In response to this, we are excited to share the news today that the U.S. Food and Drug Administration approved GENGLYCOS™ (pariglasgene breca-parvovec-opnr), a prescription gene therapy used along with nutritional management to help reduce the amount of cornstarch taken daily in adults and children 8 years and older with Glycogen Storage Disease type Ia (GSDIa)¹.

GENGLYCOS is approved under accelerated approval based on clinical study results that measured reduction in daily cornstarch intake. Verification of a clinical benefit may be needed for continued approval of GENGLYCOS.¹

Do not use GENGLYCOS if you have severe liver scarring (fibrosis or cirrhosis).

This milestone represents years of dedicated work and significant contributions from patients, families, advocacy organizations, physicians, dietitians and researchers. We are deeply grateful to the individuals living with GSDIa and their families who participated in the clinical trials and to the study investigators and staff – your dedication helped to make today possible.

We understand that patients and families will have questions about GENGLYCOS, including who is eligible and how to access the therapy. Delivering gene therapy to patients involves several essential steps and thoughtful coordination. The first step for any individual or caregiver considering treatment is to discuss with their healthcare provider whether treatment with GENGLYCOS is the right choice.

More information will be available at www.GENGLYCOS.com. Please see below for Important Safety Information.

As Ultragenyx begins this new chapter of providing this approved treatment in the U.S., our UltraCare patient services are available to support patients, families and healthcare providers throughout the treatment journey. For more information, please contact UltraCare directly at 1-888-756-8657.

We remain committed to ensuring the community has access to timely, accurate and consistent information and we will continue to work with patient organizations, such as the Association for Glycogen Storage Disease (AGSD) and the Children's Fund for GSD Research, to support these efforts.

Thank you on behalf of the Ultragenyx team,

A handwritten signature in black ink that reads "Kristin Voorhees".

Kristin Voorhees, MA
Senior Director, Patient Advocacy and Engagement

IMPORTANT SAFETY INFORMATION

What is the most important information I should know about GENGLYCOS?

- Allergic and infusion reactions, including anaphylaxis, occurred with GENGLYCOS treatment. Some of these reactions have been severe. You will be closely monitored during and after infusion for signs and symptoms including hives, flushing, low blood pressure, wheezing, trouble breathing, chest tightness, nausea, vomiting, headache, stomach pain, lightheadedness, flu-like symptoms, shivering, rash, and high blood pressure. If you think you're having a reaction to the infusion, contact your healthcare provider immediately.
- GENGLYCOS can cause certain liver enzymes to increase. You will receive oral corticosteroids with or without other medications after your infusion and will receive regular blood tests to monitor your liver function. Contact your healthcare provider right away if you experience unusual tiredness, yellowing of your skin and/or eyes, dark urine, nausea, vomiting, or pain in the upper right side of your stomach area, or if you miss a dose of your corticosteroid or vomit after taking it.
- Do not take any medicines, including herbal products or nutritional supplements, before confirming with your healthcare provider that they are not harmful to the liver.
- Talk to your healthcare provider about the possible risks of drinking alcohol following GENGLYCOS infusion.
- Problems with adrenal glands, including serious events, have occurred in patients receiving GENGLYCOS during corticosteroid treatment. Signs of adrenal gland problems can include unusual tiredness, weakness, loss of appetite, nausea, vomiting, low blood pressure, low sodium levels in the blood, and low blood sugar. A severe form called adrenal crisis can cause very low blood pressure, sudden severe stomach pain, or loss of consciousness. You will be monitored for signs and symptoms of adrenal gland problems. Do not stop taking your corticosteroid suddenly—follow your provider's instructions.
- There is a theoretical risk of tumor growth with gene therapies like GENGLYCOS; no cases have been reported in GENGLYCOS clinical studies. Contact your healthcare provider and Ultragenyx Pharmaceutical Inc. at **1-888-756-8657** if a tumor develops.

Are there any considerations for vaccination schedules and GENGLYCOS?

- Talk to your healthcare provider to determine if your vaccination schedule needs to be adjusted to accommodate corticosteroid and other medication administration prior to and after GENGLYCOS infusion.
- Vaccines should be avoided 1 month prior to GENGLYCOS administration.

What do I need to know about pregnancy and breastfeeding with GENGLYCOS?

- It is not known if GENGLYCOS can harm your baby or affect your ability to become pregnant. GENGLYCOS should not be used during pregnancy.
- It is not known if GENGLYCOS passes into breast milk. Talk to your healthcare provider about the best way to feed your baby if you take GENGLYCOS.

What do I need to know about contraception and sperm donation with GENGLYCOS?

- Women of childbearing potential should use an effective method of contraception for at least 12 months after administration of GENGLYCOS.
- For 6 months after GENGLYCOS administration, men should not donate sperm, and men capable of fathering a child and their female partner should avoid pregnancy and use an effective form of birth control.

Are there any precautions that need to be considered when handling a patient's bodily waste?

- Small amounts of GENGLYCOS can travel through the patient's blood and be temporarily passed through urine, stool, and saliva. Talk to your healthcare provider about proper hygiene when handling patient body waste. Precautions should be followed for 3 months after GENGLYCOS infusion.

What are the possible or likely side effects of GENGLYCOS?

Seven serious side effects were observed in a GENGLYCOS study, including anaphylaxis/infusion reaction (2), adrenal gland problems (2), high lactate level (2), and low blood sugar (1).

The most common side effects that occurred in individuals treated with GENGLYCOS in a study were:

- Increased liver enzymes
- Nausea
- Headache
- High triglycerides (high levels of a type of fat in the blood)
- Low adrenal hormone levels or adrenal gland problems
- Constipation
- High blood sugar
- Acne or acne-like skin rash
- Cushingoid features (changes caused by high steroid levels such as rounded face, weight gain, or increased body fat)
- Anaphylaxis (severe allergic reaction)



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These are not all the possible side effects of GENGLYCOS. Talk to your healthcare provider about any side effects that bother you or don't go away.

You are encouraged to report negative side effects of prescription drugs to the FDA.

Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Ultragenyx Pharmaceutical Inc. at 1-888-756-8657.

Please see full [Prescribing Information](#) for GENGLYCOS.

1. GENGLYCOS [Prescribing Information]. Novato, CA: Ultragenyx Pharmaceutical Inc; August 2026.